Stem Cell and Gene Therapy Agency Funds Clinical Trials Targeting Solid Tumors, and to Make Kidney Transplants Easier for Patients

Posted: November 29, 2022

South San Francisco, CA – There were more than 24,500 kidney transplants in the US last year. The procedure can be life-saving but it also requires patients to take immune suppression medications to avoid rejecting the new organ. Today the Board of the California Institute for Regenerative Medicine (CIRM) approved funding a clinical trial to test a method for performing immunosuppression-free kidney transplants.

To date CIRM has funded 85 clinical trials.

Every year California performs around 100 kidney transplants in children but, on average, around 50 of these patients will have their body reject the transplant. These children then have to undergo regular dialysis while waiting for a new organ. Even the successful transplants require a lifetime of immunosuppression medications. These medications can prevent rejection but they also increase the risk of infection, gastrointestinal disease, pancreatitis and cancer.

Dr. Alice Bertaina and her team at Stanford University were awarded $11,998,188 to test an approach that uses combined blood stem cell (HSC) and kidney transplantation with the goal to improve outcomes with kidney transplantation in children. This approach seeks to improve on the blood stem cell preparation through an immune-based purification process.

In this approach, the donor HSC are transplanted into the patient in order to prepare for the acceptance of the donor kidney once transplanted. Donor HSC give rise to cells and conditions that re-train the immune system to accept the kidney. This creates a "tolerance" to the transplanted kidney providing the opportunity to avoid long-term need for medications that suppress the immune system.

Pre-clinical data support the idea that this approach could enable the patient to stop taking any immunosuppression medications within 90 days of the surgery.

Dr. Maria T. Millan, President and CEO of CIRM, a former pediatric transplant surgeon and tolerance researcher states that "developing a way to ensure long-term success of organ transplantation by averting immune rejection while avoiding the side-effects of life-long immunosuppression medications would greatly benefit these children."

The CIRM Board also awarded $7,141,843 to Dr. Ivan King and Tachyon Therapeutics, Inc to test a drug showing promise in blocking the proliferation of cancer stem cells in solid tumors such as colorectal and gastrointestinal cancer.

Patients with late-stage colorectal cancer are typically given chemotherapy to help stop or slow down the progression of the disease. However, even with this intervention survival rates are low, usually not more than two years.

Tachyon's medication, called TACH101, is intended to target colorectal cancer (CRC) stem cells as well as the bulk tumor by blocking an enzyme called KDM4, which cancer stem cells need to grow and proliferate.

In the first phase of this trial Dr. King and his team will recruit patients with advanced or metastatic solid tumors to assess the safety of TACH101, and determine what is the safest maximum dose. In the second phase of the trial, patients with gastrointestinal tumors and colorectal cancer will be treated using the dose determined in the first phase, to determine how well the tumors respond to treatment.

The CIRM Board also awarded $5,999,919 to Dr. Natalia Gomez-Ospina and her team at Stanford University for a late-stage preclinical program targeting Severe Mucopolysaccharidosis type 1, also known as Hurler syndrome. This is an inherited condition caused by a faulty gene. Children with Hurler syndrome lack an enzyme that the body needs to digest sugar. As a result, undigested sugar molecules build up in the body, causing progressive damage to the brain, heart, and other organs. There is no effective treatment and life expectancy for many of these children is only around ten years.

Dr. Gomez-Ospina will use the patient's own blood stem cells that have been genetically edited to restore the missing enzyme. The goal

of this preclinical program is to show the team can manufacture the needed cells, to complete safety studies and to apply to the US Food and Drug Administration for an Investigational New Drug (IND), the authorization needed to begin a clinical trial in people.

Finally the Board awarded $20,401,260 to five programs as part of its Translational program. The goal of the Translational program is to support promising stem cell-based or gene projects that accelerate completion of translational stage activities necessary for advancement to clinical study or broad end use. Those can include therapeutic candidates, diagnostic methods or devices and novel tools that address critical bottlenecks in research.

The successful applicants are:

APPLICATION	TITLE	PRINCIPAL INVESTIGATOR - INSTITUTION	AMOUNT
TRAN4-14124	Cell Villages and Clinical Trial in a Dish with Pooled iPSC-CMs for Drug Discovery	Nikesh Kotecha — Greenstone Biosciences	$1,350,000
TRAN1-14003	Specific Targeting Hypoxia Metastatic Breast Tumor with Allogeneic Off-the-Shelf Anti-EGFR CAR NK Cells Expressing an ODD domain of HIF-1α	Jianhua Yu — Beckman Research Institute of City of Hope	$6,036,002
TRAN1-13983	CRISPR/Cas9-mediated gene editing of Hematopoietic stem and progenitor cells for Friedreich's ataxia	Stephanie Cherqui — University of California, San Diego	$4,846,579
TRAN1-13997	Development of a Gene Therapy for the Treatment of Pitt Hopkins Syndrome (PHS) - Translating from Animal Proof of Concept to Support Pre-IND Meeting	Allyson Berent — Mahzi Therapeutics	$4,000,000
TRAN1-13996	Overcoming resistance to standard CD19-targeted CAR T using a novel triple antigen targeted vector	William J Murphy — University of California, Davis	$4,168,679

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

Source URL: https://www.cirm.ca.gov/about-cirm/newsroom/press-releases/11292022/stem-cell-and-gene-therapy-agency-funds-clinical-trials